Harvard Conference to Focus on Personalized Medicine in Clinical Practice

As personalized medicine increasingly moves into clinical care, its potentially explosive impact on health care is the focus of this year's personalized medicine conference at Harvard University.

The conference, “Personalized Medicine: Impacting Healthcare,” is the sixth annual Harvard meeting on personalized medicine. This year’s meeting on Nov. 17-18 will explore the effect of molecular medicine on the health care delivery system as well as government, academe and business.

“The nature of health care is changing dramatically, and personalized medicine is now playing an important role in this change. It is a critical time to take stock and think about how the implementation of personalized medicine would result in better outcomes for patients in a cost-efficient manner,” said Raju Kucherlapati, Ph.D., Paul C. Cabot Professor of Genetics at Harvard Medical School and chair of the conference organizing committee.

Personalized medicine has changed some medical specialties by helping doctors understand how a patient's genetic variation will affect his or her response to a treatment. But it also has raised new questions and challenges for doctors and continued on page 2
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FDA’s promise to release new companion-diagnostics guidance before the end of the year, as well as guidance on comparative-effectiveness research. She also discussed developments in regulatory science such as collaboration among FDA offices and FDA’s goal of using data gathered during clinical trials to analyze results by subpopulation.

PMC Creates HIT Task Force
PMC has formed a Health Information Technology (HIT) Task Force that is working with Darrell West, Vice President, Governance Studies and Director, Center for Technology Innovation, Brookings Institution, to issue a Brookings white paper this winter. PMC believes the paper is a critical opportunity to inform policymakers how HIT can facilitate the advancement of personalized medicine and improve health care. The paper will be released at a public seminar at the Brookings Institution in January.

House Panel Looks into Consumer Genetic Tests
On July 22, the House Energy and Commerce Subcommittee on Oversight and Investigations held a hearing on “Direct-To-Consumer Genetic Testing and the Consequences to the Public Health.” Among the issues lawmakers raised: the validity of the tests, the unintended consequences of test misinterpretation or incorrect results, and patients’ privacy. The hearing followed a GAO investigation on such tests. Vance Vanier, M.D., Navigenics’ President and Chief Executive Officer, told the subcommittee that the tests are “probabilistic, not predictive.”

Personalized Treatments Are Increasingly Part of Health Care

researchers. A panel on new therapies will feature K. Peter Hirth, Ph.D., Founder and Chief Executive Officer of Plexxikon, whose anti-melanoma targeted therapy PLX4032 was featured in a New York Times article about cousins who participated in a randomized controlled clinical trial for the drug. The cousin who received PLX4032 is still alive; the other, who received the control therapy, died.

With molecular diagnostics increasingly used in medicine, one panel will discuss their role in diseases including hypertrophic cardiomyopathy, a hypertrophy of the heart muscle, and adult macular degeneration, which can cause blindness.

In a separate panel, David Parker, Ph.D., vice president of Boston Healthcare, will discuss reimbursement issues for these tests in conjunction with the release of a paper on the subject commissioned by the Personalized Medicine Coalition, which helped plan the conference and will present its sixth annual award for leadership in personalized medicine to William Dalton, Ph.D., M.D., CEO of the Moffitt Cancer Center.

The first keynote speaker will be Mikael Dolsten, M.D., Ph.D., president of Pfizer Worldwide Research and Development. Pharmaceutical companies are increasingly investing in molecular diagnostics to help guide therapy choices. According to Pfizer Chairman and CEO Jeff Kindler, “The changes we’re making recognize the end of the era of ‘blockbusters’—medicines that meet widespread primary health care needs and therefore generate billions in revenues. To be sure, we are still pursuing blockbusters, but we are also focusing on addressing many specialized needs of many smaller groups of people. This personalized medicine approach means new opportunities for people who are sick, for physicians and for our shareholders.”

Tom Ashbrook of National Public Radio’s “On Point” will discuss the conference on a live half-hour broadcast on Nov. 18 that will feature an interview with FDA Commissioner Margaret Hamburg, M.D.

The conference, a collaboration of the Partners HealthCare Center for Personalized Genetic Medicine, Harvard Medical School and Harvard Business School, will take place at Joseph B. Martin Conference Center, 77 Avenue Louis Pasteur, Boston. To register, visit www.personalizedmedicineconference.org.
Scrubtity Drives Policymakers’ New Interest in Personalized Medicine

BY AMY MILLER | PUBLIC POLICY DIRECTOR

Policymakers’ interest in personalized medicine reached a crescendo this summer when a Republican Congressman described consumer genomics as “snake oil” during a congressional hearing on the matter. But while consumer genomics companies were generating a lot of buzz, several interesting policy developments for personalized medicine were taking shape behind the scenes in Congress and at the Food and Drug Administration (FDA).

In Congress, Representative Patrick Kennedy, D-R.I., partnered with Representative Anna Eshoo, D-Calif., to reintroduce the Genomics and Personalized Medicine Act (H.R. 5440). Their new bill keeps some of the original provisions of the bill, such as funding for research and training grants, and also adds new provisions, including PMC’s suggestion to codify the current Health and Human Services personalized health care initiative. Unfortunately, financial incentives, which would encourage investment in personalized medicine products, were again left out.

The number of members of Congress interested in personalized medicine continues to grow. With such increased interest, it is conceivable that we can get some good provisions enacted in law. With the 111th Congress wrapping up, several senators have invited us to articulate ways to improve the bill for the 112th Congress and to propose a set of financial incentives that will advance personalized medicine. We have convened a work group to compile PMC’s suggested updates; please contact me if you would like to participate.

One stumbling block for personalized medicine is the lack of clarity regarding FDA’s role in regulating laboratory developed tests (LDTs). The FDA contends that it possesses the authority to regulate LDTs but does not exercise it unless necessary. Thus, when FDA does regulate particular lab services, it does so in what seems to some to be a haphazard and disruptive way.

Two efforts over the summer sought to address this stumbling block. First, draft legislation circulated Washington that would develop a new office or center within the FDA. The entity would have clear jurisdiction over diagnostics, would be shaped by a stakeholder-engaged comment and rulemaking process, and would establish a risk-based approach to regulation. In other words, the level of regulation envisioned would be correlated with the intended use of the test.

Second, FDA recognized the diagnostic industry’s discomfort with the current state of play. To address concerns that proposed solutions be developed with stakeholder input, FDA hosted a two-day listening session on the regulation of LDTs. Many PMC members submitted public comments, which are available at http://www.regulations.gov. (see box on page 5).

PMC will continue to track developments and work with policymakers to propose solutions to the policy barriers facing personalized medicine. To assist us, the Brookings Institution will issue a white paper, Enabling Personalized Medicine through Health Information Technology, at a public seminar in January. PMC will also publish an issue brief next month on the reimbursement challenges personalized medicine diagnostics encounter at the Centers for Medicare and Medicaid Services.

Several senators have invited us to articulate ways to improve the Genomics and Personalized Medicine Act for the 112th Congress and to propose a set of financial incentives that will advance personalized medicine.
FDA Moves to Establish New Regulatory Framework for Personalized Medicine

As personalized medicine has developed through the first decade of the 21st century, the Food and Drug Administration has faced numerous challenges to refashion a regulatory system that was designed for a one-size-fits-all era and didn’t really advance personalized medicine products.

Now, with the agency increasing vigilance over direct-to-consumer sales of genomic tests and nearing completion of new regulations, FDA is building a new oversight framework for personalized medicine.

On October 6, the agency issued a white paper, “Advancing Regulatory Science for Public Health,” which outlines the regulatory system it has designed to take advantage of recent breakthroughs in science and technology. According to the report, these breakthroughs “have the potential to prevent, diagnose and treat disease.”

Specifically, FDA wants to invest $25 million to develop new strategies that will improve the agency’s ability to promote and approve personalized medicine products, pointing to its work with the National Institutes of Health on a trial to tailor future breast cancer treatments based on the molecular profile of specific tumors.

In a recent opinion editorial in the New England Journal of Medicine, NIH Director Francis Collins, M.D., Ph.D., and FDA Commissioner Margaret Hamburg, M.D., wrote, “Researchers have discovered hundreds of genes that harbor variations contributing to human illness, identified genetic variability in patients’ responses to dozens of treatments, and begun to target the molecular causes of some diseases. In addition, scientists are developing and using diagnostic tests based on genetics or other molecular mechanisms to better predict patients’ responses to targeted therapy. The challenge is to deliver the benefits of this work to patients.”

Already, FDA plans to define a number of key pieces of personalized medicine’s regulatory puzzle: draft guidance on the qualification process for biomarkers has been released, and new rules are expected soon for laboratory-developed tests (LDTs).

The biomarker guidance informs developers of the criteria FDA uses to vet the utility of biomarkers and evaluate clinical trial data in targeted drug development.
The FDA currently regulates diagnostic tests, manufactured by one company and then sold as a kit to a laboratory for genetic testing, but not LDTs, which are tests developed and performed by a laboratory. (The laboratories that provide LDTs are regulated by the Centers for Medicare and Medicaid Services, but the tests themselves are not.)

It is less clear whether draft guidance on co-development of diagnostic and therapeutic products will be released by year-end, as Dr. Hamburg promised in her speech to the Personalized Medicine Coalition earlier this year.

However, remarks by FDA officials at recent conferences indicate that the agency has made substantial progress in deciding how to treat companion diagnostics, diagnostics that are required or recommended when selecting or dosing a particular therapy.

Meanwhile, the forthcoming LDT rules may provide the biggest window on FDA’s thinking. Officials have said that they are moving away from regulating some types of tests differently from others, as they had once planned to do for IVDMIAs. In fact, all diagnostics could be brought under the same regime, an FDA official told attendees at the agency’s July hearing on LDTs.

As LDTs and test kits have become more similar, the need to regulate them separately “has faded,” said the official, Elizabeth Mansfield, Ph.D., the director for personalized medicine at the Office of In Vitro Diagnostic Device Evaluation and Safety at FDA’s Center for Devices and Radiological Health (CDRH). “A risk-based framework might be appropriate for all manufacturers and add value in laboratory developed tests as well as commercially distributed diagnostics,” she said.

Meanwhile, she added, FDA is considering loosening rules for some diagnostics that are now required to meet premarket approval before their release.

“We may look at them and say that premarket review doesn’t add much value, let’s not do that,” Dr. Mansfield said. Instead, she added, the agency will redirect its resources to higher-risk tests.

But even before the new regulations are released, in July FDA began to restrict consumers’ direct access to DNA test kits. After consumer-genomics company Pathway Genomics attempted to sell saliva sample-collection kits for its online genetic risk assessments through retail pharmacy stores Walgreens and CVS, FDA sent a warning letter to Pathway, and followed up with letters to a number of other consumer-genomics companies.

And last month, FDA announced a pilot program with CMS to jointly approve some diagnostic tests. Because CMS decisions on test reimbursement tend to influence private insurers, a positive review could enable a company to quickly receive widespread reimbursement and diffusion of a new test. (A negative review might have the opposite impact.) Companies may choose whether or not to participate in the pilot program.

Until the new LDT rules are released, the agency has advised test makers to consult its existing guidance documents, request face-to-face meetings with FDA, and attend the agency’s informational sessions if they have questions about how to proceed with a formal submission for a particular test.

Excerpts from FDA Hearing on Laboratory Developed Tests

Since a July hearing on planned laboratory-developed test (LDT) regulations, FDA has received more than 100 public submissions on the subject. A sampling follows:

Rep. Louise Slaughter, D-N.Y.: “I encourage HHS and the FDA to enhance the oversight of genetic tests through enhanced regulations and guidance documents.”

Abbott: “Many tests that can provide important medical information for patient care simply cannot sustain the sort of resource investment currently required for IVD approval. Possible solutions are streamlined pathways (for all classes of products), better defined exemptions for rarer diseases, and alternatives to encourage submissions as IVD devices. One approach which should be strongly considered is a pathway for approval of analytical claims only which would not require submission of patient outcomes data.”

American Clinical Laboratory Association: “It is important to emphasize the breadth, scope and value of LDTs, to demonstrate the need to move the oversight of LDTs forward in a measured way that does not have unintended consequences for patient care.”

Genomic Health: Recommends “clear, predictable, non-duplicative oversight, recognition of existing CLIA and state oversight of laboratory operations, additional oversight of tests already in use.”

National Health Council: “We urge the FDA to give special consideration to LDTs that focus on rare diseases, just as it does for therapies that target rare diseases. Under such an approach, the FDA’s regulatory requirements would vary depending on the degree of risk of the diagnostic tests.”

FDA

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Personalized Medicine Coalition 2010 Membership

Organizations new to the Coalition are underlined

AGENCY PARTICIPANTS
Centers for Disease Control and Prevention
Centers for Medicare and Medicaid Services
National Cancer Institute
U.S. Food and Drug Administration

CLINICAL LABORATORY TESTING SERVICES
Genelex Corporation
Iversen Genetic Diagnostics, Inc.
Laboratory Corporation of America (LabCorp)
Laboratory for Personalized Molecular Medicine
Pathway Genomics Corporation
Quest Diagnostics

CONSUMER GENETIC TESTING SERVICES
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American Clinical Laboratory Association
Association of Medical Diagnostics Manufacturers (AMDM)
BIO (Biotechnology Industry Organization)
PhRMA

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CTX, Inc.
HP Health and Life Sciences
IBM Healthcare and Life Sciences
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Millennium: The Takeda Oncology Company
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PATIENT ADVOCACY GROUPS
Alliance for Aging Research
Genomix Canada
Hypertrophic Cardiomyopathy Association-HCMA
National Alliance for Hispanic Health
National Brain Tumor Society

RESEARCH & EDUCATIONAL INSTITUTIONS
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American Society of Human Genetics (ASHG)
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Children’s Hospital & Research Center Oakland
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College of American Pathologists
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The Critical Path Institute (C-Path)
Duke University
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Institute of Genomic Medicine, University of Miami
Marshall Clinic
Mayo Clinic
National Foundation for Cancer Research
National Jewish Health
National Pharmaceutical Council
The Ohio State University Medical Center
Partners HealthCare Center for Personalized Genetic Medicine
Poliambulatorio Euganea Medica
RTI International
Scripps Research Institute
Shady Grove Adventist Hospital
Translational Genomics Research Institute (TGen)
United States Diagnostic Standards (USDS)
University of Rochester
University of Utah
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Personalized Medicine Partners, LLC
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PricewaterhouseCoopers LLP
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Technic Solutions, LLC
Townsend and Townsend and Crew LLP
Valerie August & Associates, LLC – Biotechnology Recruiter
William Blair & Company
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VENTURE CAPITAL
Burlir & Company
Kleiner Perkins Caufield & Byers
Lemhi Ventures
MDV-Mohr Davidow Ventures
Pappas Ventures
Third Rock Ventures, LLC
Reimbursement of Personalized Medicine Diagnostics

BY DAVID PARKER, PH.D., VICE PRESIDENT, BOSTON HEALTHCARE

As medicine and our understanding of human biology and genetics have become more sophisticated in recent decades, it has become increasingly clear that individualized aspects of patients cause them to manifest diseases differently and to respond differently—in terms of both efficacy and safety—to medical treatments. The resulting uncertainty about how an individual might respond to a particular treatment regimen has significant adverse consequences for the quality and cost of health care: quality is degraded for those patients who do not exhibit positive treatment responses, while the cost of their failed treatment is wasted, and the overall cost of successful treatment often is increased by the delay in getting the right therapy. Clearly then, the personalization of medicine offers the potential for increasing quality while decreasing cost, by better defining an individual’s disease process and permitting the more appropriate targeting of treatment.

By their nature, diagnostic tests play a central role in the personalization of medicine: one can only better characterize a disease process, or predict who might respond well or poorly to a treatment, by measuring some biological characteristic of the patient. In fact, the explosion of human genetic information and advances in diagnostic technology platforms over the past decade have at last permitted real progress in personalized medicine. During this time, we have seen the development and validation of personalized medicine diagnostic (PMD) tests that examine individualized differences and correlate the results with meaningful differences in disease likelihood and course, as well as treatment suitability.

However, despite the dramatic growth in the number and power of advanced diagnostic technologies, the increasing clinical and economic value of the tests derived from them, and the therapeutic power of the drugs whose use can and should be guided by those tests, the reimbursement system for diagnostic tests has not evolved to accommodate the development and adoption of PMDs. Reimbursement for diagnostic tests is grounded in decisions made and systems developed decades ago. It reflects outmoded patterns of health care delivery and increasingly antiquated payment mechanisms. It relies on timelines that progress far more slowly than the pace of PMD development, evidence standards that are ill suited to the clinical and economic realities of PMD development, and payment methodologies that reflect neither the purpose nor the clinical and economic value of PMDs.

Among the stumbling blocks this system poses for PMDs are:

• a system of descriptive test codes designed to report simple methods or individual analytes, but with no mechanisms to reflect clinical results or values, or to describe complex tests with advanced methodologies and/or multiple analytes;

• a process for coverage determination that has few standards, varies widely from test to test, and may depend on robust outcomes evidence that can be prohibitively expensive as well as impractical or impossible to gather, and

• a payment system anchored in the costs of tests developed decades ago, which is applied variably to novel diagnostic assays.

Consequently, many PMDs are not reimbursed appropriately. Such inappropriate reimbursement inevitably leads to inadequate access. If there is uncertainty about the ability to recoup the cost of developing or performing a PMD test, then the laboratory will not offer it, if indeed anyone even invests in its development. If the physician must provide an elaborate justification of medical necessity, then the test will not be ordered. If the patient is told that Medicare is unlikely to reimburse, then the test will be refused. In all cases, the patient will be denied the benefits of personalized treatment—the right medicine, at the right time, for the right indication.

What, then, can be done to improve this situation? Because appropriate reimbursement results from a complex interplay of codes, coverage decisions, policies, and payment amounts, addressing any of these aspects individually is inadequate. Instead, obtaining the full benefits of PMDs will require a fundamentally different reimbursement paradigm. This paradigm will require a system of PMD coding capable of distinguishing among PMDs with subtly different technical or clinical characteristics. It will require a payment methodology capable of delivering payment commensurate with the clinical and health economic value provided by PMDs. And it will require a coverage process and evidence standards compatible with the realities of the clinical uses and market sizes of PMDs.

How we move from the current state to this new paradigm is not yet clear. We may need to carve PMDs out of the current reimbursement system and give responsibility for aspects of their reimbursement to new or different authorities. Novel methods for projecting clinical utility on the basis of small clinical data sets may need to be developed. Ultimately, legislation may be required to address deficiencies in Medicare’s reimbursement of PMDs.

What is clear is this: if we want more PMDs, the required investment in product and clinical development must be recoverable through appropriate reimbursement, and reimbursement uncertainty must be reduced. It is critical that we address these issues, and soon, if we are to see personalized medicine continue to advance, and enjoy its benefits.

The views expressed in this article are those of the author and do not necessarily represent the opinions of the Personalized Medicine Coalition. The article draws on Dr. Parker’s forthcoming issue brief for PMC titled, “The Adverse Impact of the U.S. Reimbursement System on the Development and Adoption of Personalized Medicine Diagnostics.”
Duke Seeks to Catalyze Use of Personalized Medicine

In an ambitious move to more quickly incorporate molecular medicine in the health care system, officials at Duke are creating a new personalized medicine center and a company to expedite the development of biomarkers, in addition to other interdisciplinary entities to expedite both discovery and clinical application in the field of personalized medicine.

“At Duke we are pioneering genomic approaches to treating diseases such as cancer and diabetes, and building models for incorporating genomic medicine into clinical care,” said Victor Dzau, M.D., Chancellor for Health Affairs, Duke University, and CEO, Duke University Health System. “By transforming medicine from reacting to disease to proactively preventing illness, we will ultimately reduce costs while improving care.”

“Our core strategy is to create a rapid learning model for personalized medicine,” said Geoffrey Ginsburg, M.D., Ph.D., who will head a new Center for Personalized Medicine at Duke. “We want the Center to serve as a catalyst to accelerate the incorporation of personalized medicine within the health system.”

The Center for Personalized Medicine will bring the latest research in genomics, genetics and informatics to three separate clinical practices based at Duke that together serve a total of 35,000 patients. Its website, http://dukepersonalizedmedicine.org, will focus on sharing information across multiple fields, said Dr. Ginsburg, who is currently Director of the Center for Genomic Medicine at the Duke Institute for Genomic Science and Public Policy, and a PMC Board member.

Lack of interest in funding the expensive and often unproductive translational research that is necessary to enable doctors to use a new discovery means that many years can elapse between discovery and medical application. The period has been termed the “valley of death” because patients who might have been helped by a new therapy or device die before it becomes common medical practice. Although lack of translational research affects medicine broadly, the problem is particularly acute in personalized medicine, which relies on integrating both new therapies and diagnostics.

By integrating work from several fields into clinical care, the Center for Personalized Medicine will tackle multiple problems that have plagued personalized medicine in its move from bench to bedside. Among these are physicians’ lack of training in interpreting genomics data for patients and the delay in bringing new findings to patients. Some studies show it can take 12 to 17 years before medical breakthroughs are routinely used by 80 to 90 percent of health care providers.

“We are really trying to reduce translational time,” Dr. Ginsburg said. Dr. Ginsburg said the Center will use primary-care and specialty practice environments to conduct implementation research and define health care delivery models. It will determine how personalized medicine is best integrated into practice, workflow, and what skill sets, training and informatics are needed to deploy new tools. In addition to capturing clinical and behavioral data, the Center will conduct comparative effectiveness research.

Among the Center’s initial projects are:

• integrating selected genetic testing to identify populations at high risk for chronic disease;
• researching changes in behavior in patients in response to information on genetic variants associated with risks for type 2 diabetes and heart attacks;
• educating physicians on pharmacogenomic tests—their use and steps for incorporating them into practice, and
• studying the effectiveness of current disease risk analysis.

Meanwhile, Duke is partnering with companies in an effort to expedite the development of discoveries related to molecular medicine, spurred in part by
its researchers’ discovery last year of the genomic basis for some patients’ failure to respond to treatment for hepatitis C, one of the most common infections in the world.

The groundbreaking discovery was helped by the fact that Duke integrated genotyping and analysis into an industry-sponsored clinical trial, according to Victoria Christian, Chief Operating Officer of the Duke Translational Research Institute, who spearheaded the creation of a corporate partnership with LabCorp called the Biomarker Factory.

By creating the Biomarker Factory, Duke hopes to replicate its success with hepatitis C. The partners hope to bring biomarkers from discovery to clinical use in record time through their combination of talents. At the end of the discovery chain, LabCorp stands ready to commercialize any usable discoveries, Ms. Christian said.

“It’s a very systematic approach to running through the huge numbers of clinical samples that Duke generates every year,” said Andrew Conrad, Ph.D., chief scientific officer for LabCorp. “They’re the raw materials for looking at a biomarker. So it’s an enormously efficient process.”

Dr. Conrad said new discoveries will facilitate a change in public attitudes toward personalized medicine. “Everybody’s mindset is going to switch,” he said. “They’ll say these biomarkers are a good way to help people.”

The Factory has received 30 biomarker applications and is in the process of selecting eight for its first round of development, he said.

The partnership with LabCorp is only the beginning of Duke’s opportunities, said Terry Walker, Ph.D., General Manager of the Biomarker Factory. “We think it probably will lead to some new companies,” he said. “Some cases will go straight to LabCorp. In others there will be development of freestanding niche. In other cases, there are plenty of drugs out there that are poorly understood, which hobbled effective marketing.”

Duke has already inked another agreement with CancerGuide Diagnostics to develop oncology-specific applications. Under the agreement, CancerGuide gains exclusive commercial rights to a portfolio of validated molecular signatures that predict patient response to certain therapeutics, as well as access to other new molecular discoveries.

Ultimately, Dr. Ginsburg said, Duke believes it’s the right time to make the transformational change promised by personalized medicine. “At the end of the day, we want to see no difference between ‘personalized medicine’ and ‘Duke medicine,’” he said. “Five to ten years from now, we hope there will no longer be any need for a catalytic center.”

Elizabeth Schwinn

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**Therapeutic Drug Discovery Pipeline**

- **NIH** $29 Billion/Yr
- **Translation Gap - the “Valley of Death”**
- **Biotech & Pharma** $64 Billion/Yr

Sources: Michael J. Fox Foundation for Parkinson’s Research; FasterCures
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cold water on personalized medicine, “A Decade Later, Genetic Map Yields Few New Cures.”

“Scientific discoveries have always been separated from their clinical contributions, not by 10 years, but usually by 25 years or more,” Leon Rosenberg, a molecular biologist at Princeton University and Huntington Willard, director of the Duke University Institute for Genome Sciences and Policy, pointed out in a letter to the editor of The New York Times following its article and a subsequent editorial that made the same point. More important, however, is the fact that new diagnostic products and targeted therapies, based on genetic and other molecular diagnoses, are indeed coming on the market at a very rapid clip. PMC is at pains to bring attention to these breakthroughs; they are transforming medical practice, especially in oncology but in other areas as well.

Proponents of personalized medicine argue that the pace of progress is conditioned not only by our understanding of the underlying biology but also by what Lee Hood, M.D., Ph.D., President of the Institute of Systems Biology and the inventor of the DNA sequencer, calls a “people problem:” our inability so far to align regulatory, reimbursement and educational systems to produce more effective therapies at lower costs.

Personalized medicine advocates were encouraged by the election of Barack Obama. They had hoped that President Obama, the original author of the Genomics and Personalized Medicine Act, whose purpose was to put the prestige and power of the United States government behind the new paradigm, would at least call attention to personalized medicine, if not promote it by creating a friendlier landscape for its development. Unfortunately that did not happen, the new president’s plate being rather full.

Significantly, this is beginning to change.

On June 28, Margaret Hamburg, M.D., Commissioner of the U.S. Food and Drug Administration, and Francis Collins, M.D., Ph.D., Director of the U.S. National Institutes of Health, published an important article in the New England Journal of Medicine entitled “The Path to Personalized Medicine.” In it, these two senior architects of U.S. government to advance translational and regulatory science

“We intend to help make personalized medicine a reality.”

— Margaret Hamburg, M.D. and Francis Collins M.D., Ph.D.

guidance on the co-development of diagnostic and therapeutic products. And, in a nod to reducing the increasing cost of pharmaceutical research and development, they envision more efficient clinical trials that focus on subgroups of patients with specific biomarkers.

To be sure, Drs. Hamburg and Collins do not propose creating financial incentives that would lead to more investment in personalized medicine. Nor do they point out the need to ensure that reimbursement for personalized medicine products does not become a final, even higher hurdle —after the science and the regulatory barriers are overcome—that stymies the development of personalized medicine. It is good that NIH and FDA are coordinated; it would be better if the Centers for Medicare and Medicaid Services also shared their commitment to advance personalized medicine.

Following on the heels of “The Path to Personalized Medicine,” on Aug. 24, Vice President Biden issued a report on how the American Recovery and Reinvestment Act is transforming the American economy through innovation. Among other things, the report notes that due to public investment in gene sequencing, the cost to map one’s personal genome is likely to fall below one thousand dollars within five years, and thereby “unlock cures and insights into some of the most debilitating diseases existing today.”

A bolder statement embracing personalized medicine could not be imagined.
The Personalized Medicine Coalition has elected Stephen Eck, M.D., Ph.D., Vice President for Translational Medicine & Pharmacogenomics at Eli Lilly & Company, to its Board of Directors.

Dr. Eck is a board-certified hematologist/oncologist with broad drug development experience in oncology and neuroscience. At Lilly, he is responsible for the clinical pharmacology components of drug development including both early phase clinical studies and late stage drug development studies. His group also develops the biomarkers and companion diagnostics needed for effective decision-making and for tailoring therapeutics to the right patient population.

“As Stephen Eck is a leader who has made a real difference in advancing the concepts of personalized medicine in drug development through the research he and his teams conduct,” said Wayne Rosenkrans, PMC’s chairman. “His knowledge and skills will further our efforts to promote the incorporation of targeted therapies into medical care.”

“Lilly has a broad interest in researching and developing innovative new medicines tailored to improve the outcomes of individual patients with cancer, diabetes, neurologic disorders, and mental health conditions, as well as cardiovascular and other metabolic diseases,” Dr. Eck said. “As such, I look forward to working with the Personalized Medicine Coalition to help advance innovative therapies.”

A key part of Dr. Eck’s work at Lilly is conducted in the Diagnostic and Experimental Medicine Group and the Laboratory for Experimental Medicine. In addition, the Pharmacogenomics group is an important part of Translational Medicine and leads the effort to develop the right medicine for the right patient.

Dr. Eck serves on the Scientific Advisory Board of the Alliance for Cancer Gene Therapy and on the Executive Committee of the Biomarkers Consortium. The Biomarkers Consortium is managed by the Foundation of the National Institutes of Health (NIH). Members include NIH, the Food and Drug Administration, the Pharmaceutical Research and Manufacturers of America, and the Biotechnology Industry Organization.

PMC Announces New Board Member

Stephen Eck, M.D., Ph.D.
Doctors and payers still need to be convinced that personalized medicine will transform health care, according to the Fall 2010 issue of *Biotechnology Healthcare*. But a partnership between Ohio State University Medical Center and the Institute for Systems Biology may soon provide some data. “We are the employer, payer and provider of health care to our employee base,” says PMC board member Clay Marsh, M.D., director of OSUMC’s Center for Personalized Health Care. “This gives us an opportunity to test whether different types of approaches to health care and wellness-based care can lower costs and improve outcomes.”

In a key development in health care reform implementation, the Government Accountability Office announced its selection of the Patient-Centered Outcomes Research Institute Board of Governors in late September. *GenomeWeb* quoted PMC’s statement on the selection of Pfizer’s Freda Lewis-Hall, M.D., one of PMC’s nominees, praising her “commitment to and knowledge of both comparative effectiveness research and personalized medicine.” Meanwhile, *Inside Health Policy* questioned GAO’s logic in failing to include PMC nominee Janet Woodcock, M.D., or another representative from FDA.

PMC President Edward Abrahams warned against the possibility that comparative effectiveness research will turn into a “blunt instrument that smothers the opportunity to improve care and reduce costs” in a letter to the editor published by *The New York Times* in September.

The FDA continued to reevaluate its oversight of laboratory developed tests, with additional focus on consumer genetic tests at a two-day meeting in July, *Drug Topics* reported in September. The meeting included a panel featuring PMC Chairman Wayne Rosenkrans, as well as representatives from 23andMe and Navigenics.

The personalized medicine approach will pay off in the face of critical challenges, including “regulation, reimbursement, and education,” PMC President Edward Abrahams noted in September’s issue of *BioPhotonics*.

PMC President Edward Abrahams applauded the inclusion of the principles of personalized medicine in the health reform law’s language on comparative effectiveness research, specifically that the research must take into account results from studies’ subpopulations, as reported by *CQ HealthBeat News* in August.

Jeff Balser, M.D., Ph.D., dean of Vanderbilt University School of Medicine, told *The Tennessean* that the school is working to move medicine from “the best care on average to the best care for the individual.” PMC President Edward Abrahams applauded Vanderbilt in the article as well as Harvard, Ohio State University Medical Center, Brown University, Baylor College of Medicine and Duke University for their leadership in tailoring treatments to patients.

The American Cancer Society’s journal *Cancer* noted the significance of health care reform on cancer treatments, including PMC’s statement on the policy victories won in the legislation in August.

The Biotechnology Industry Organization, PhRMA, and the Personalized Medicine Coalition each nominated advocates of personalized medicine from Pfizer, Amgen, and GlaxoSmithKline for the governing board of the Patient-Centered Outcomes Research Institute, reported *FDA Week* in July.

In an op-ed in *PharmExec.com*, Suri Harris of CAHG quoted Edward Abrahams’ statement in support of health reform: “Policymakers can now turn their attention to the issues surrounding medical innovation, improved patient care, and reduced systemic costs.”

This year marked the tenth anniversary of the announcement of the near completion of the Human Genome Project, *Drug Topics* reported in June. PMC President Edward Abrahams told the magazine that given the developments of the past ten years, “Personalized medicine will have enormous ramifications for pharmacists.” Significant progress has already been made in incorporating pharmacogenomics into treatment, he said, and in the future, there will be a growing need for molecular diagnostics before a drug is prescribed.

The use of genetic profiling to scan for diseases is increasing, said *The San Diego Union-Tribune* in June. PMC President Edward Abrahams said, “Science points out that individuals are different, and one size does not fit all when it comes to medicine, but science alone isn’t going to drive the pace of innovation. How we regulate, reimburse and educate will all make a difference.”